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Human Medicines Development and Evaluation

Committee for Orphan Medicinal Products (COMP) meeting report on the review of applications for orphan designation

September 2012

The Committee for Orphan Medicinal Products held its 137th plenary meeting on 4-5 September 2012. During the meeting the COMP elected for a 3-year mandate the new Chair, Prof. Bruno Sepodes and the new Vice-Chair, Ms Lesley Greene.

Orphan medicinal product designation

The COMP adopted 12 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

1. Opinions adopted at the second COMP discussion, following the sponsor's response to the COMP list of questions:

- **[2-Cyano-3-cyclopropyl-3-hydroxy-N-(3-methyl-4-trifluoromethylphenyl)prop-2-enamide]** for treatment of traumatic spinal cord injury, Algiax Pharmaceuticals GmbH.
- **Asp-Arg-Val-Try-Ile-His-Pro** for treatment of acute lung injury, Tarix Pharmaceuticals Limited.
- **Mavoglurant** for treatment of fragile X syndrome, Novartis Europharm Limited.
- **Rucaparib** for treatment of ovarian cancer, Clovis Oncology UK Limited.

2. Opinions adopted at the first COMP discussion:

- **Alpha-1 proteinase inhibitor (for inhalation use)** for treatment of cystic fibrosis, Grifols Deutschland GmbH[1].
- **Belinostat** for treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated), TopoTarget A/S.
- **Humanised monoclonal IgG4 antibody against tissue factor pathway inhibitor** for treatment of haemophilia A, Novo Nordisk A/S[2].

[1] Adopted via written procedure on 12 September 2012
[2] Adopted via written procedure on 12 September 2012

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

- **Lurbinectedin** for treatment of ovarian cancer, Pharma Mar SA Sociedad Unipersonal.
- **Mixture of two allogeneic human pancreatic cancer cell lines stably transduced with a retroviral vector encoding the murine *alpha-(1,3)-galactosyltransferase* gene** for treatment of pancreatic cancer, European Medical Advisory Services Limited.
- **Obinutuzumab** for treatment of chronic lymphocytic leukaemia, Roche Registration Limited[3].
- **Recombinant human lecithin cholesterol acyltransferase** for treatment of lecithin cholesterol acyltransferase deficiency, Alphacore Pharma Limited[4].
- **Liposomal daunorubicin** for treatment of acute myeloid leukaemia, Galen Limited

Public summaries of opinions will be available on the EMA website following adoption of the respective decisions on orphan designation by the European Commission.

Other information on the orphan medicinal product designation

Lists of questions

The COMP adopted 8 lists of questions on initial applications. These applications will be discussed again at the next COMP meeting prior to the adoption of an opinion.

Oral hearings

8 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 4 applications for orphan medicinal product designation were withdrawn.

Detailed information on the orphan designation procedure

An overview of orphan designation procedures since 2000 is provided in Annex 1.

The list of medicinal products for which decisions on orphan designation[5] have been given by the European Commission since the last COMP meeting is provided in Annex 2.

Applications for marketing authorisation for orphan medicinal products

Details of those designated orphan medicinal products that have been subject of a new European Union marketing authorisation application through the centralised procedure since the last COMP plenary meeting are provided in Annex 3.

Details on the opinions for marketing authorisation for orphan medicinal products adopted by the Committee for Medicinal Products for Human Use (CHMP) can be found in the CHMP meeting reports on the EMA website: http://www.ema.europa.eu/ema/index.jsp?curl=pages/about_us/general/general_content_000508.jsp&mid=WC0b01ac0580028d2a.

[3] Adopted via written procedure on 12 September 2012
[4] Adopted via written procedure on 12 September 2012
[5] Details of all orphan designations granted to date by the European Commission are entered in the Community Register of Orphan Medicinal Products http://ec.europa.eu/health/documents/community-register/html/index_en.htm

Article 5 (12) of Regulation (EC) No 141/2000 of the European Parliament and of the Council

In line with its responsibility to review whether or not a designated orphan medicinal product still fulfils the designation criteria prior to the granting of a marketing authorisation, the COMP adopted 3 opinions recommending to the European Commission that the following orphan medicinal products be kept in the EU registry of orphan medicinal products:

- **Adcetris** (monoclonal antibody against human CD30 covalently linked to the cytotoxin monomethylauristatin E) for treatment of anaplastic large cell lymphoma; Takeda Global Research and Development Centre (Europe) Ltd.
- **Adcetris** (monoclonal antibody against human CD30 covalently linked to the cytotoxin monomethylauristatin E) for treatment of Hodgkin lymphoma; Takeda Global Research and Development Centre (Europe) Ltd.
- **Glybera** (adeno-associated viral vector expressing lipoprotein lipase) for treatment of lipoprotein lipase deficiency; uniQure biopharma B.V.

Upcoming meetings

- The 138th meeting of the COMP will be held on 3-5 October 2012.

Other matters

The main topics addressed during the meeting related to:

- 4 Protocol Assistance letters were adopted.

Note

This monthly report, together with other information on the work of the European Medicines Agency, can be found on the EMA website: www.ema.europa.eu

Contact our press officer

Monika Benstetter or Sabine Haubenreisser

Tel. +44 (0)20 7418 8427

E-mail: press@ema.europa.eu

Annex 1

Overview for orphan medicinal product designation procedure since 2000

Year	Applications submitted	Applications discussed in reporting year	Positive COMP opinions	Applications withdrawn	Final negative COMP opinions	EC designations	Orphan medicinal products authorised
2012	136	126	95 (75%)	30 (24%)	1 (1%)	102	4
2011	166	158	111 (70%)	45 (29%)	2 (1%)	107	5
2010	174	176	123 (70%)	51 (29%)	2 (1%)	128	4
2009	164	137	113 (83%)	23 (17%)	0[6] (0%)	106	9
2008	119	118	86 (73%)	31 (26%)	1 (1%)	73	6
2007	125	117	97 (83%)	19 (16%)	1 (1%)	98	13
2006	104	103	81 (79%)	20 (19%)	2 (2%)	80	9
2005	118	118	88 (75%)	30 (25%)	0 (0%)	88	4
2004	108	101	75 (74%)	22 (22%)	4 (4%)	73	6
2003	87	96	54 (56%)	41 (43%)	1 (1%)	55	5
2002	80	76	43 (57%)	30 (40%)	2[7] (3%)	49	4
2001	83	92	64 (70%)	27 (29%)	1 (1%)	64	3
2000	72	32	26 (81%)	6 (19%)	0 (0%)	14	0
Total	**1536**	**1450**	**1056 (73%)**	**375(26%)**	**17 (1%)**	**1037**	**72**

[6] Negative opinion adopted in 2009 was revised and finalised in 2010, therefore it is not included in 2009 listing
[7] Following a quality assurance exercise it was identified that this figure needed correction

Annex 2

Medicinal products granted a European Union designation as orphan medicinal product by the European Commission since the July 2012 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
(2S)-2-{[(2R)-2-[({[3,3-dibutyl-7-(methylthio)-1,1-dioxido-5-phenyl-2,3,4,5-tetrahydro-1,2,5-benzothiadiazepin-8-yl]oxy}acetyl)amino]-2-(4-hydroxyphenyl)acetyl]amino}butanoic acid	Treatment of progressive familial intrahepatic cholestasis	Albireo AB	13 June 2012	17 July 2012
(2S)-2-{[(2R)-2-[({[3,3-dibutyl-7-(methylthio)-1,1-dioxido-5-phenyl-2,3,4,5-tetrahydro- 1,2,5-benzothiadiazepin-8-yl]oxy}acetyl)amino]-2-(4-hydroxyphenyl)acetyl]amino}butanoic acid	Treatment of Alagille syndrome	Albireo AB	11 July 2012	9 August 2012
(2S)-2-{[(2R)-2-[({[3,3-dibutyl-7-(methylthio)-1,1-dioxido-5-phenyl-2,3,4,5-tetrahydro- 1,2,5-benzothiadiazepin-8-yl]oxy}acetyl)amino]-2-(4-hydroxyphenyl)acetyl]amino}butanoic acid	Treatment of primary biliary cirrhosis	Albireo AB	11 July 2012	9 August 2012
1-[(2-Chloro-4-methoxyphenoxy)methyl]-4-[(2,6-dichlorophenoxy)methyl]benzene	Prevention of poliomyelitis in patients with immunodeficiencies deemed at risk	ProPhase Development Ltd.	13 June 2012	17 July 2012
Covalently closed DNA plasmids coding for cytomegalovirus *phosphoprotein 65* and *glycoprotein B* genes	Prevention of cytomegalovirus disease in patients with impaired cell mediated immunity deemed at risk	Astellas Pharma Europe B.V.		
Elotuzumab	Treatment of multiple myeloma	Bristol-Myers Squibb Pharma EEIG	11 July 2012	9 August 2012
Hexasodium phytate	Treatment of calciphylaxis	Sanifit Laboratoris, S.L.	13 June 2012	17 July 2012
Human apotransferrin	Treatment of congenital hypotransferrinaemia	Sanquin Blood Supply Foundation	13 June 2012	17 July 2012

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Humanised monoclonal antibody against epidermal growth factor receptor	Treatment of glioma	Abbott Laboratories	11 July 2012	9 August 2012
Humanised monoclonal antibody against P-selectin	Treatment of sickle cell disease	Quintiles Ireland Ltd	11 July 2012	9 August 2012
Ketoconazole	Treatment of Cushing's syndrome	Agenzia Industrie Difesa-Stabilimento Chimico Farmaceutico Militare	11 July 2012	9 August 2012
Metreleptin	Treatment of Barraquer-Simons syndrome	Aptiv Solutions (UK) Limited	13 June 2012	17 July 2012
Metreleptin	Treatment of Berardinelli-Seip syndrome	Aptiv Solutions (UK) Limited	13 June 2012	17 July 2012
Metreleptin	Treatment of familial partial lipodystrophy	Aptiv Solutions (UK) Limited	13 June 2012	17 July 2012
Metreleptin	Treatment of Lawrence syndrome	Aptiv Solutions (UK) Limited	13 June 2012	17 July 2012
N-Butyldeoxygalactonojirimycin	Treatment of Fabry disease	Actelion Registration Limited	11 July 2012	9 August 2012
Recombinant anti-CD3-bi-single-chain-Fv-diphtheria toxin fusion protein	Treatment of cutaneous T-cell lymphoma	AOP Orphan Pharmaceuticals AG	11 July 2012	9 August 2012
Recombinant anti-CD3-bi-single-chain-Fv-diphtheria toxin fusion protein	Treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated)	AOP Orphan Pharmaceuticals AG	11 July 2012	9 August 2012
Recombinant human monoclonal antibody against activin receptor type IIB	Treatment of inclusion body myositis	Novartis Europharm Limited	11 July 2012	9 August 2012
Recombinant human pentraxin-	Treatment of idiopathic pulmonary fibrosis	Appletree Europe S.à.r.l.	13 June 2012	17 July 2012
Vatreptacog alfa (activated)	Treatment of haemophilia A	Novo Nordisk A/S	11 July 2012	9 August 2012
Vatreptacog alfa (activated)	Treatment of haemophilia B	Novo Nordisk A/S	11 July 2012	9 August 2012

Annex 3

Designated orphan medicinal products that have been subject of a new European Union marketing authorisation application under the centralised procedure since the July 2012 COMP monthly report

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
(-)-17(cyclopropylmethyl)-1,14 ß-dihydroxy-4,5 alpha-epoxy-6ß-[N-methyl-trans-3-(3-furyl) acrylamido] morphinan hydrochloride	Winfuran	Toray International U.K. Limited	EU/3/02/115	Treatment of uremic pruritus
N-(methyl-diazacyclohexyl-methylbenzamide)-azaphenyl-aminothiopyrrole	Masican	AB Science	EU/3/04/251	Treatment of malignant gastrointestinal stromal tumours